

**STATEMENT OF**  
**CONGRESSMAN HENRY A. WAXMAN**  
**CHAIRMAN, SUBCOMMITTEE ON HEALTH AND THE ENVIRONMENT**  
**PRESS CONFERENCE OF THE**  
**NATIONAL COMMISSION ON ORPHAN DISEASES**  
**APRIL 12, 1989**

IN THE INTRODUCTION TO THE COMMISSION'S REPORT, THERE IS A QUOTE OF A PHYSICIAN WHO TREATS PATIENTS WITH RARE DISEASES. THE DOCTOR CAPTURED THE SPIRIT OF THIS COMMISSION, AND OF THE CONGRESS WHEN WE CREATED THE COMMISSION IN 1985.

HE SAID: "WE ALL SHARE EQUALLY IN THE RISK OF HAVING A CHILD WITH A GENETIC DISEASE. THEREFORE, ALL OF US OUGHT TO SHARE IN PROVIDING EFFECTIVE TREATMENT TO THE UNFORTUNATE CHILDREN AFFECTED BY THEM."

THIS REPORT IS ABOUT OUR GOVERNMENT'S RESPONSIBILITY TO THOSE INDIVIDUALS AND FAMILIES AFFECTED BY RARE DISEASES. THE CONGRESS, IN CALLING FOR THE REPORT, AND THE COMMISSION, IN DEVELOPING IT, HAVE HEARD THE VOICE OF THE AMERICAN PEOPLE LOUD AND CLEAR. THEY BELIEVE THAT THE UNLUCKY FEW WHO SUFFER WITH A RARE DISEASE DESERVE AS MUCH ATTENTION AND HELP FROM THE GOVERNMENT AS DO THE MANY WHO HAVE MORE COMMON ILLNESSES.

THE GENESIS OF THE COMMISSION CAN BE FOUND IN THE CONGRESSIONAL DELIBERATIONS INVOLVING THE ORPHAN DRUG ACT. FOR YEARS WE STRUGGLED TO UNDERSTAND HOW TO FASHION LEGISLATION THAT WOULD ENCOURAGE THE DEVELOPMENT OF ORPHAN DRUGS. BY THE TIME WE FINALLY SUCCEEDED, WE HAD LEARNED FIRST HAND THAT DRUG DEVELOPMENT IS BUT ONE SMALL PART OF THE PAINFUL REALITY OF RARE DISEASES.

WE LEARNED THERE ARE NO ANSWERS FOR MANY RARE DISEASES, AND FOR THOSE WHO SUFFER WITH THESE DISEASES, THE LACK OF KNOWLEDGE CAN BE DEVASTATING. WE LEARNED THAT EVEN WHEN IMPORTANT AND USEFUL INFORMATION IS AVAILABLE, OFTEN IT IS NOT EFFECTIVELY COMMUNICATED TO PRACTICING PHYSICIANS WHO ARE RESPONSIBLE FOR DIAGNOSING AND TREATING PATIENTS.

CONGRESS NEEDED EXPERT ADVICE. WE NEEDED A COMPREHENSIVE REVIEW OF PUBLIC AND PRIVATE EFFORTS AND A PLAN FOR ADDRESSING THE NEEDS, ALL THE NEEDS, OF PEOPLE WHO SUFFER WITH RARE DISEASES. SO CONGRESS CREATED THE NATIONAL COMMISSION ON ORPHAN DISEASES. TODAY, WE ARE GETTING EXACTLY WHAT WE WANTED.

DR. THOENE, TO YOU AND THE OTHER COMMISSION MEMBERS AND STAFF, LET ME SAY CONGRATULATIONS AND THANK YOU. YOUR UNDERTAKING WAS ENORMOUS. YOU HAVE BEEN DEDICATED AND HARDWORKING. THIS REPORT IS A BLUEPRINT FOR HOW THE FEDERAL GOVERNMENT CAN FULFILL ITS RESPONSIBILITY TO THOSE WHO SUFFER WITH RARE DISEASES.

THE COMMISSION HAS GIVEN CONGRESS AND THE ADMINISTRATION A GREAT DEAL TO CONSIDER. I AM CONFIDENT THE REPORT CONTAINS THE INFORMATION AND THE IDEAS WE NEED. AFTER CAREFUL REVIEW OF THE REPORT, I INTEND TO INTRODUCE THE NECESSARY LEGISLATION TO IMPROVE THE CONDITIONS FOR RARE DISEASE RESEARCH AND FOR THE PREVENTION, DIAGNOSIS AND TREATMENT OF RARE DISEASES.

IN 1983, WITH THE PASSAGE OF THE ORPHAN DRUG ACT, THE CONGRESS AND THE REAGAN ADMINISTRATION TOOK THE FIRST IMPORTANT STEP IN HELPING THE MILLIONS OF AMERICANS WHO SUFFER WITH RARE DISEASES. NOW, IT IS TIME FOR THE CONGRESS AND THE BUSH ADMINISTRATION TO WORK TOGETHER TO ADDRESS THE OTHER TRAGIC CIRCUMSTANCES THAT AFFECT THE LIVES OF SO MANY CITIZENS.